

### **Diabetes Fact Sheet**

CIRM funds many projects seeking to better understand diabetes and to translate those discoveries into new therapies.

### Description

Diabetes comes in two forms: type 1 (also known as juvenile) and type 2 (also known as adult). As many as 3 million people in the U.S. have type 1 diabetes, which is the form primarily being targeted by stem cell research.

Type 1 diabetes is an autoimmune disorder where the body's own immune system destroys the cells in the pancreas that make insulin. Insulin normally circulates in the bloodstream after a meal and allows cells of the body to take up sugar and use it for food. Without insulin, cells starve and the sugar builds up in the bloodstream where it can damage the kidneys, blood vessels and retina.

Any potential cure for type 1 diabetes requires replacing the lost insulin-producing cells of the pancreas. Currently, the only cells that can be used for such a transplant come from donated organs, which are in short supply. Such insulin-producing cell transplants are also risky because the cells can be rejected by the recipient's body if they don't receive immune suppressing drugs.

To solve the first problem, groups of CIRM-funded researchers have developed methods to make replacement insulin-producing cells derived from human embryonic stem cells, which can be grown in large amounts. Implanted into mice and rats these cells are able to regulate blood sugar.

To get around the problem of rejection, CIRM-funded teams have placed cells in a device that implants under the skin and shields the cells from the immune system. Other groups are studying how to regulate the immune system to make stem cell-derived transplants safer.

### **Clinical Stage Programs**

### Caladrius Biosciences

Caladrius is targeting the immune system as an alternative strategy for treating patients with type 1 diabetes. This disease causes the immune system to destroy the insulin-producing cells of the pancreas. The team is developing a stem cell-based therapy using the patient's own cells. They will take cells, called regulatory T cells (Tregs), from the patient's own immune system, expand the number of those cells in the lab and enhance them to make them more effective at preventing the autoimmune attack on the insulin-producing cells.

- Read more about the project
- Read more about the clinical trial

### ViaCyte

The team has developed a way of maturing embryonic stem cells into an early form of the pancreatic cells that produce insulin. They then insert those cells into a permeable device that can be implanted under the skin. Inside the device, the cells mature into insulin-producing cells and the hope is that they will help maintain blood sugar levels at a healthy level. The team is currently testing the safety and efficacy of this device in a Phase 1 clinical trial.

- Read more about this project
- Read more about the clinical trial

# Progress and Promise in Diabetes Research

# **CIRM Grants Targeting Diabetes**

| Researcher<br>name   | Institution   | Grant Title   | Grant Type  | Approved funds |
|----------------------|---|---|---|----------------|
| Mark<br>Anderson     | University of<br>California, San<br>Francisco           | Generation of a functional thymus to induce immune tolerance to stem cell derivatives   | Basic Biology V                                   | \$1,191,000    |
| Douglas<br>Losordo   | Caladrius<br>Biosciences                                | Phase 2 Safety and Efficacy Study of CLBS03 Autologous<br>T-Regulatory Cells in Adolescents with Recent Onset<br>Type 1 Diabetes Mellitus | Clinical Trial Stage<br>Projects                  | \$12,211,255   |
| David<br>Tirrell     | California Institute of<br>Technology                   | Engineered matrices for control of lineage commitment in human pancreatic stem cells  | Basic Biology V                                   | \$526,896      |
| Esther<br>Latres     | JDRF International                                      | JDRF Encapsulation Consortium Fall 2017 Meeting   | Conference II                                     | \$42,425       |
| Jeffrey<br>Bluestone | University of<br>California, San<br>Francisco           | A CIRM Disease Team for the Treatment and Cure of Diabetes  | Disease Team<br>Planning                          | \$55,000       |
| Howard<br>Foyt       | ViaCyte, Inc.   | Clinical trial of directly vascularized islet cell replacement therapy for high-risk type 1 diabetes                                      | Clinical Trial Stage<br>Projects                  | \$19,752,463   |
| Howard<br>Foyt       | ViaCyte, Inc.   | Clinical Development of a Cell Therapy for Diabetes   | Accelerated<br>Development<br>Pathway I           | \$8,783,852    |
| Howard<br>Foyt       | ViaCyte, Inc.   | Clinical trial of directly vascularized islet cell replacement therapy for high-risk type 1 diabetes                                      | Clinical Trial Stage<br>Projects                  | \$19,752,463   |
| Catriona<br>Jamieson | University of<br>California, San Diego                  | Alpha Stem Cell Clinic for the Development of Regenerative Therapies  | Alpha Stem Cell<br>Clinics                        | \$7.999,137    |
| Mark<br>Walters      | Children's Hospital<br>of Oakland Research<br>Institute | University of California, San Francisco (UCSF) CIRM Alpha<br>Stem Cell Clinic   | Alpha Stem Cell<br>Clinics Network<br>Expansion   | \$7,999,999    |
| Roslyn<br>Isseroff   | University of<br>California, Davis                      | Scaffold for dermal regeneration containing pre-<br>conditioned mesenchymal stem cells to heal chronic<br>diabetic wounds                 | Preclinical<br>Development<br>Awards              | \$4,620,144    |
| Yang Xu              | University of<br>California San Diego                   | Development of immune invisible beta cells as a cell therapy for type 1 diabetes through genetic modification of hESCs                    | Quest - Discovery<br>Stage Research<br>Projects   | \$2,167,200    |
| Felicia<br>Pagliuca  | Semma<br>Therapeutics                                   | Personalized Cell Therapy for Diabetes  | Therapeutic<br>Translational<br>Research Projects | \$597,333      |

| Alan<br>Agulnick   | ViaCyte, Inc.                                  | Preclinical development of an immune evasive islet cell replacement therapy for type 1 diabetes   | Quest - Discovery<br>Stage Research<br>Projects     | \$1,470,987  |
|--------------------|--|---|---|--------------|
| Senta<br>Georgia   | Children's Hospital<br>of Los Angeles          | Developing a personalized approach to beta cell replacement for patients with a genetic form of diabetes                                  | Inception -<br>Discovery Stage<br>Research Projects | \$180,000    |
| Shuvo Roy          | University of<br>California, San<br>Francisco  | Silicon Nanopore Membrane encapsulated enriched-<br>Beta Clusters for Type 1 Diabetes treatment   | Quest - Discovery<br>Stage Research<br>Projects     | \$1,113,000  |
| Allan<br>Robins    | ViaCyte, Inc.                                  | Cell Therapy for Diabetes   | Disease Team<br>Research I                          | \$22,999,933 |
| Kevin<br>D'Amour   | ViaCyte, Inc.                                  | Stem cell-derived islet cell replacement therapy with immunosuppression for high-risk type 1 diabetes                                     | Late Stage<br>Preclinical Projects                  | \$3,544,721  |
| Peter Butler       | Semma<br>Therapeutics                          | Personalized Cell Therapy for Diabetes  | Therapeutic<br>Translational<br>Research Projects   | \$4,230,186  |
| Didier<br>Stainier | University of<br>California, San<br>Francisco  | Endodermal differentiation of human ES cells  | SEED Grant  | \$611,027    |
| Tejal Desai        | University of<br>California, San<br>Francisco  | Thin Film Encapsulation Devices for Human Stem Cell derived Insulin Producing Cells   | Quest - Discovery<br>Stage Research<br>Projects     | \$1,092,063  |
| Yang Xu            | University of<br>California, San Diego         | Developing induced pluripotent stem cells into human therapeutics and disease models  | Early Translational I                               | \$5,165,028  |
| Julie<br>Sneddon   | University of<br>California, San<br>Francisco  | Designing a cellular niche for transplantation of human embryonic stem cell-derived beta cells  | Quest - Discovery<br>Stage Research<br>Projects     | \$2,006,076  |
| Olivia Kelly       | ViaCyte, Inc.                                  | Methods for detection and elimination of residual human embryonic stem cells in a differentiated cell product                             | Early Translational I                               | \$5,405,397  |
| Fouad<br>Kandeel   | City of Hope,<br>Beckman Research<br>Institute | 2009 Rachmiel Levine Diabetes and Obesity Symposium:<br>Advances in Diabetes Biology, Immunology and Cell<br>Therapy                      | Conference  | \$15,000     |
| Evert Kroon        | ViaCyte, Inc.                                  | Development of the Theracyte Cellular Encapsulation<br>System for Delivery of human ES Cell-derived Pancreatic<br>Islets and Progenitors. | Tools and<br>Technologies I                         | \$827,072    |
| Fouad<br>Kandeel   | City of Hope,<br>Beckman Research<br>Institute | 2013 Rachmiel Diabetes and Obesity Levine Symposium -<br>Advances in Diabetes Research  | Conference  | \$27,750     |
| Jeffrey            | University of<br>California, San               | Stem cell tolerance through the use of engineered antigen-specific regulatory T cells   | Transplantation<br>Immunology                       | \$1,152,768  |

| Fouad<br>Kandeel | City of Hope,<br>Beckman Research<br>Institute | 2011 Rachmiel Levine Diabetes and Obesity Symposium:<br>Advances in Diabetes Research   | Conference            | \$15,000    |
|------------------|--|---|-----------------------|-------------|
| Charles<br>King  | University of<br>California, San Diego         | Biological relevance of microRNAs in hESC differentiation to endocrine pancreas   | Basic Biology III     | \$1,313,649 |
| Maike<br>Sander  | University of<br>California, San Diego         | Deciphering transcriptional control of pancreatic beta-<br>cell maturation in vitro   | Basic Biology IV      | \$1,258,560 |
| Fouad<br>Kandeel | City of Hope,<br>Beckman Research<br>Institute | 2012 Rachmiel Levine Diabetes and Obesity Symposium:<br>Advances in Diabetes Research   | Conference            | \$15,000    |
| Howard<br>Foyt   | ViaCyte, Inc.                                  | Preclinical and clinical testing of a stem cell-based combination product for insulin-dependent diabetes                      | Strategic Partnership | \$9,475,070 |
| Richard<br>Jove  | City of Hope,<br>Beckman Research<br>Institute | Innovation and Translational Stem Cell Therapy for Diabetes and Neurological Diseases: Paving the way for real life solutions | Conference            | \$14,878    |
|                  |  |   |                       |             |

# **CIRM Diabetes Videos**



Diabetes: Progress toward stem cell therapies, a Live



Ron Piran, Sanford-Burnham - CIRM Stem Cell #SciencePitch



Eugene Brandon, Viacyte, Inc. - CIRM Stem Cell #SciencePitch



Diabetes: Progress and Promise in Stem Cell Research



Diabetes: Advancing Stem Cell Therapies - 2011 CIRM Grantee Meeting



Alan Lewis Talks About Getting an **Embryonic Stem Cell-Based Therapy** to Patients



Spotlight on Type 1 Diabetes: **Welcoming Remarks** 



Spotlight on Type 1 Diabetes: Seminar by Ed Baetge, Ph.D.



Spotlight on Type 1 Diabetes: Seminar by Peter Butler, M.D.



Spotlight on Type 1 Diabetes: Seminar by Joelle Johnson



Stem Cell Based Diabetes Treatment: Alan Lewis - CIRM Science Writer's Seminar

# **News and Information**

- CIRMResearch blog entries on type 1 diabetes research progress
- CIRM Loan to Boost Diabetes Research (San Diego Business Journal)

- Diabetes Health Guide (New York Times)
- San Diego company studies stem cell implant as a Type 1 diabetes treatment (Los Angeles Times)

### Resources

- NIH: Diabetes Information
- CDC: Diabetes Resources
- Find a clinical trial near you: NIH Clinical Trials database
- Juvenile Diabetes Research Foundation
- American Diabetes Association
- Stem Cell Netword diabetes page
- Family Caregiver Alliance
- National Family Caregivers Association

## Find Out More:

Stem Cell FAQ | Stem Cell Videos | What We Fund

Source URL: https://www.cirm.ca.gov/our-progress/disease-information/diabetes-fact-sheet